

March ___, 2021

The Honorable Rosa DeLauro
Chairwoman, Labor, Health and Human
Services Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

The Honorable Tom Cole
Ranking Member, Labor, Health and Human
Services Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

The Honorable Betty McCollum
Chairwoman, Defense Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

The Honorable Ken Calvert
Ranking Member, Defense Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

The Honorable Sanford Bishop Jr.
Chairman, Agriculture, Rural Development,
Food and Drug Administration Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

The Honorable Jeff Fortenberry
Ranking Member, Agriculture, Rural Development,
Food and Drug Administration Subcommittee
Committee on Appropriations
U.S. House of Representatives
Washington, DC 20515

Dear Chairs DeLauro, McCollum, Bishop and Ranking Members Cole, Calvert, and Fortenberry:

Thank you for your continued strong support of ALS (amyotrophic lateral sclerosis) research. Your support for ALS research through the Department of Defense's (DOD) ALS Research Program (ALSRP), the National Institutes of Health (NIH), the Centers for Disease Control and Prevention's (CDC) National ALS Registry, and the Food and Drug Administration's (FDA) Orphan Products Grants Program has been instrumental in advancing our understanding of ALS. We believe that increases in funding for these programs, tempered by rigorous evaluation and accountability, is critical to finding new treatments and a cure for ALS. With your help, we can make a meaningful improvement in the course of this disease, providing hope to every American family facing ALS.

As you know, ALS is a fatal neurodegenerative disease that destroys a person's ability to control muscle movement. As the disease progresses, people become trapped inside a body they can no longer control. The average life expectancy for a person living with ALS is just two to five years after diagnosis with no cure.

The prognosis of people diagnosed with ALS is the same as it was nearly 80 years ago when baseball legend Lou Gehrig lost his fight with the disease. However, with your support, we can make a meaningful improvement in the course of this disease, providing hope to every American family facing ALS. To achieve this goal, Congress must increase federal funding for ALS research, including funds to increase the number of ALS clinical trials.

Labor, Health and Human Services Subcommittee

National Academy of Sciences

We request at least \$1 million for a National Academies of Sciences, Engineering, and Medicine (NASEM) study to develop a plan and policy recommendations to make ALS a livable disease within a decade. A NASEM study is important because it will provide the government and disease-space advocates with a blueprint to achieve this goal. Accompanying this programmatic request, we request the report language below:

HHS, Office of the Secretary

The bill includes an additional \$1 million for the Secretary to commission an independent study by the National Academies of Sciences, Engineering, and Medicine (NASEM) to identify and recommend actions for the public, private, and not-for-profit sectors to undertake to make amyotrophic lateral sclerosis (ALS) a livable disease within a decade. Given the significant adverse physical, financial, psychological impact this progressive neurodegenerative disease has on the individuals and families affected by it, a comprehensive assessment of what is necessary to address its effects is warranted. The study should include, but not be limited to: how to develop more effective and meaningful treatments and a cure; interventions to reduce and prevent the progression and complications of ALS; the type and range of care and services people and families with ALS need and how to ensure they receive comprehensive, quality care; what care, services, and preventive measures people at-risk of ALS need; and how to improve the quality-of-life, health, and well-being of affected individuals and families. The study should be submitted to Congress by the end of FY 2024.

National Institutes of Health

We request at least \$130 million for ALS research at NIH – a \$20 million increase over FY2021. Increased funding for ALS research at NIH is important because the agency is the largest funder of ALS research in the world, giving it the best chance to attract the next generation of scientists, accelerate discovery and development of new treatments and increase the number of ALS clinical trials. We also request the report language below to organize a trans-agency initiative to develop an ALS research strategic plan:

National Institutes of Health, Office of the Director

ALS Research Coordination and Acceleration – The Committee is aware of the significant need to expand scientific understanding of amyotrophic lateral sclerosis (ALS) and to translate ALS science more rapidly into effective treatments that can make ALS a livable disease. In order to achieve these outcomes as soon as possible, the Committee directs the NIH to organize a trans-agency initiative to develop an ALS research strategic plan. The plan, which should be developed in collaboration with the nation's leading ALS patient and biomedical research organizations, should: identify the most promising areas of research and the specific NIH activities where additional funding could lead to more rapid translation of discoveries for

treatments, prevention, and interventions or technologies that can reduce the burden of ALS; identify which institutes are undertaking ALS and ALS-related research and which are not but have a role to play; and uncover any impediments to ALS research. As part of this effort, the agency should hold at least one public meeting at which stakeholders can provide testimony. This effort should include, but not be limited to: the National Institute of Neurological Disorders and Stroke; the National Institute on Aging; National Institute of Environmental Health Sciences, the National Institute of Mental Health; the National Human Genome Research Institute; the National Institute of Arthritis and Musculoskeletal and Skin Diseases, and, the National Center for Advancing Translational Sciences.

CDC National ALS Registry and Biorepository:

We request \$10 million for the National ALS Registry and biorepository. Continued funding for the Registry and biorepository is needed because the program is uniquely positioned to identify risk factors for ALS to reduce the number of new cases. Not only does the program fund research on disease etiology and risk factors, it also makes all data available to researchers, connects patients with clinical trials, and conducts surveillance of incidence and prevalence. We also request the report language below to increase transparency on incidence and prevalence reporting and how the Registry's findings can be translated into human applications that can reduce the number of new ALS cases.

*Centers for Disease Control and Prevention, Environmental Health
Within 90 days, present a plan to Congress to change the oversight and advisory process for the National ALS Registry to increase transparency and better reflect the needs of the ALS community. This plan should consider ways to translate Registry findings to human application that can lower the risks and incidence of ALS, make incidence and prevalence reporting faster, more accurate, and more inclusive, and include a range of estimates rather than point estimates of ALS prevalence. Equally important are updates to the survey process to ensure the data they collect are impactful and respectful of participant burden. Finally, the plan should include collaboration with the Office of Management and Budget to ensure that the ALS community can view as much Registry information as possible without compromising the privacy of participants.*

Defense Subcommittee:

DOD ALS Research Program

We request \$60 million for the ALS Research Program (ALSRP) – a \$20 million increase over FY2021. Studies funded by the DOD, VA and NIH have found that people who served in the military are up to twice as likely to develop and die from ALS. We believe it continues to be important for the DOD to identify and research all diseases that may be related to service in the U.S. military, including ALS.

Increased funding for ALSRP will allow the program to build on a solid foundation of promising preclinical research, which currently represents the scope of the program's portfolio. ALSRP is uniquely positioned to expand its portfolio into early phase clinical trials to bridge the so-called "valley of death" between promising preclinical research and human studies in ALS drug development. With several promising ALSRP-funded preclinical projects nearing early phase clinical trials, now is the time to increase investment in the program. We request report language below to expand ALSRP's investments in early phase clinical trials to increase the number of ALS clinical trials that represent the treatments and cures of tomorrow:

Peer-Reviewed ALS Research

The Committee is aware of promising research underway through the Amyotrophic Lateral Sclerosis Research Program (ALSRP). Given that people who served in the military are up to twice as likely to develop and die from ALS as those with no history of military service, it is especially important this progress be continued into early phase clinical trials. The Committee encourages the Department of Defense to pursue clinical research that can bring effective treatments to people living with ALS as soon as possible.

Agriculture Subcommittee:

FDA Orphan Products Grants Program

We request at least \$50 million for the Orphan Products Grants Program (OPGP) at FDA which can fund ALS clinical trials and invest in regulatory science to speed the approval of ALS treatments. OPGP is an incentive program that has been supporting clinical trial research since 1983 but has only been able to support six ALS trials to date. It is important to increase the number of ALS clinical trials because there is no cure for ALS and too few clinical trials. Providing the FDA with the ability to fund more of their own ALS clinical trials and apply their own unique regulatory expertise in those ALS trials will expedite treatment development, foster innovative trial designs that complement and speed the FDA regulatory processes, and enable natural history studies to more quickly understand ALS progression and pathology.

Conclusion

We appreciate your consideration of our FY2022 appropriations requests for ALS research. With these strategic investments, we can increase the number of clinical trials and reduce the number of new ALS cases. Combined, this investment will help us change the trajectory for this disease and bring more effective treatments to every American with ALS.

Sincerely,